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Aplastic Anemia (short version)

Recommendations from the society for diagnosis and therapy of haematological and oncological diseases

DGHO DEUTSCHE GESELLSCHAFT FÜR HÄMATOLOGIE UND MEDIZINISCHE ONKOLOGIE

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Aplastic Anemia (short version)

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Compliance rules:

- Guideline
- Conflict of interests

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1 Summary

Aplastic anemia (AA) (synonyms: panmyelopathy, panmyelophthisis) comprises a heterogeneous group of rare diseases leading to bone marrow insufficiency. The more common acquired aplastic anemias are to be distinguished from the 'inherited bone marrow failure syndromes'. Clinically, aplastic anemia is dominated by the symptoms of bi- or tricytopenia with anemia, neutropenia, thrombocytopenia in various combinations and to variable degrees.

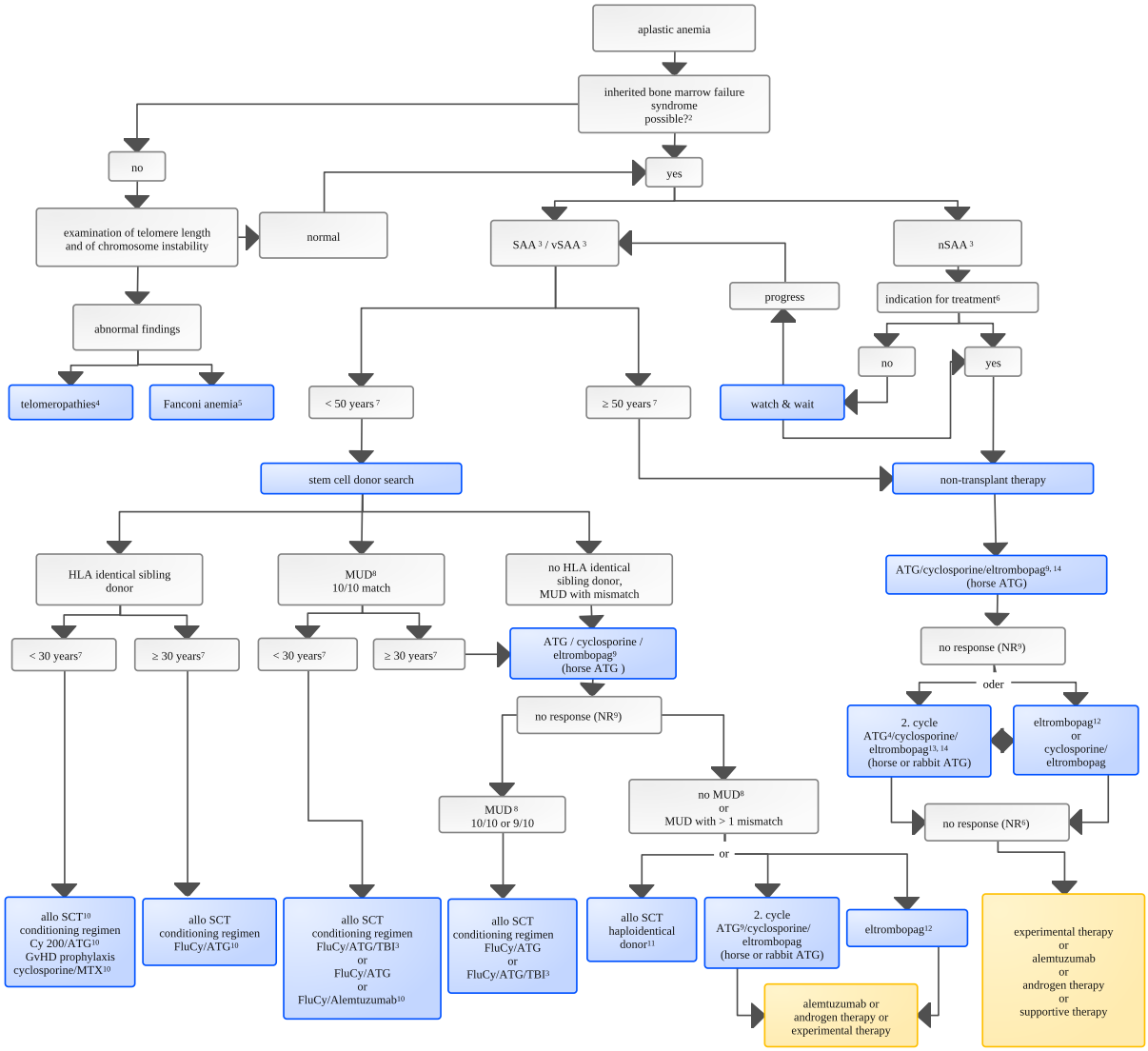
Therapy is based on the etiology and especially on the clinical manifestation. In cases of non-severe/moderate aplastic anemia (nSAA/MAA), wait-and-see approach is recommended or, if treatment is indicated, immunosuppressive therapy. In severe or very severe aplastic anemia, allogeneic stem cell transplantation is curative. If not feasible, immunosuppressive therapy with horse anti-thymocyte globulin (ATG) plus ciclosporin A (CSA) is the gold standard in first-line treatment in these cases. New drugs such as the thrombopoietin receptor eltrombopag expand the therapeutic options.

As aplastic anemia is a rare disease and often requires further therapy optimization. Contact should be made with an expert center before initiating therapy to clarify, among other things, whether treatment is possible in the context of a clinical trial.

2 Therapy

The current treatment algorithm is depicted in Figure 1.

Figure 1: Therapy algorithm for aplastic anemia



Legend:

curative treatment intent; non-curative treatment intent;

blue: established therapy, yellow: experimental therapy

[1] recommendations for children in protocols and guidelines of the Pediatric Study Group Aplastic Anemia;

[2] see chapter 6. 3 (Link to German guideline)

[3] nSAA - non-severe AA (aplastic anemia); SAA – severe AA, vSAA - very severe AA

[4] see chapter 6. 3. 1 (Link to German guideline)

[5] see chapter 6. 3. 2 (Link to German guideline)

[6] see chapter 6. 1. 1 (Link to German guideline)

[7] important is the biological age and not the age in years; this is important for patients ≥50 years, who do not respond to non-transplant therapy

[8] MUD – matched unrelated donor; see chapter 6.1.3.2.1 (Link to German guideline)

[9] first line standard therapy for patients with vSAA / SAA, who are ineligible for allogeneic stem cell transplantation is the combination of ATG
(anti-thymocyte globulin), ciclosporine A and eltrombopag. (Eltrombopag is not approved for this indication in Germany).

[10] allo SCT: allogeneic stem cell transplantation; Cy200 - cyclophosphamide 200 mg/kg, FluCy/ATG – fludarabine, low dose cyclophosphamide and ATG; MTX – methotrexate, TBI – total body irradiation

[11] allo SCT with haploidentical stem cell donor; see chapter 6.1.3.2.1 (Link to German guideline)

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16 Disclosures

Conflicts of interest can be found in the full German version of the guideline.